4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0001]

Developing and Using Precision Therapies in the "Omics" Era: Generating and Interpreting Evidence for Rare Subsets; Public Workshop

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is announcing a public workshop entitled "Developing and Using Precision Therapies in the 'Omics' Era: Generating and Interpreting Evidence for Rare Subsets." This public workshop is being cosponsored with the Center for Translational and Regulatory Sciences at the University of Virginia (UVA). The goals of this public workshop are to facilitate discussion on current scientific approaches using rare subsets during drug development programs and to further seek input from multiple stakeholders on approaches to obtain evidence that inform the regulatory evaluation of therapeutic products in rare subsets of patients identified through in-vitro diagnostic testing when specific, controlled trials are not feasible.

DATES: The public workshop will be held on December 12, 2014, from 9 a.m. to 5 p.m. Individuals who wish to attend the public workshop in person or via a live Webcast must register online by December 1, 2014, at: https://www.signup4.net/Public/ap.aspx?OID=130&EID=DEVE96E. Section II of this document provides attendance and registration information.

ADDRESSES: The public workshop will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (rm. 1503A), Silver Spring, MD 20993-0002. Entrance for the public workshop participants (non-FDA employees) is through Building 1 where routine security check procedures will be performed. For parking and security information, please refer to http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

FOR FURTHER INFORMATION CONTACT: Padmaja Mummaneni, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 2164, Silver Spring, MD 20993-0002, 301-796-2027, email: padmaja.mummaneni@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Therapeutic products are increasingly targeted to patients who have molecular characteristics that are diagnostic of a particular subtype of disease, prognostic for better or worse outcomes, or predictive of treatment response. The advent of next-generation sequencing and other high throughput technologies has enabled the development of in-vitro diagnostic tests that are able to detect rare molecular variations, specifically in the patient, tumor, or microbial DNA sequence. FDA and UVA are cosponsoring an open public workshop among stakeholders in the pharmaceutical industry, representatives from academia, regulatory scientists, and other interested parties on the development and usage of diagnostic and therapeutic products that respectively have the potential to identify and treat patients with rare molecular characteristics. It is important for regulatory agencies, pharmaceutical and diagnostic industries, and the medical

community, including payers, to have a mutual understanding of various forms of evidence that could inform regulatory and medical decision making. The public workshop will help identify key components of such an evidence framework when therapeutic effectiveness is being evaluated in patients with molecular characteristics that are rare or have not been studied in clinical trials.

II. Attendance and Registration

The FDA Conference Center at the White Oak location is a Federal facility with security procedures and limited seating. Individuals who wish to attend the public workshop must register on or before December 1, 2014, by visiting: https://www.signup4.net/Public/ap.aspx?OID=130&EID=DEVE96E.

Early registration is recommended. Registration is free and will be on a first-come, first-served basis. However, FDA may limit the number of participants from each organization based on space limitations. On-site registration on the day of the public workshop will be based on space availability.

FDA will provide additional background information at the time the Federal Register notice is published and an agenda approximately 2 weeks before the public workshop at the FDA Meeting Information page, which is available online at: http://wcms.fda.gov/FDAgov/Drugs/NewsEvents/ucm416622.htm?SSContributor=true.

If you need special accommodations because of disability, please contact Padmaja Mummaneni (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the public workshop.

A live Webcast of this public workshop will be viewable on Adobe Connect at https://collaboration.fda.gov/rsw2014/ on the day of the public workshop.

Dated: November 17, 2014.

Leslie Kux,

Associate Commissioner for Policy.

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